The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.

Grant Award Details

The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.

Grant Type: Alpha Stem Cell Clinics

Grant Number: AC1-07659

Project Objective: The Objective of the Alpha Clinics program is to support high quality stem cell clinical trials and to create accelerating and value add resources (AVARs) to accelerate and improve stem cell research.

Investigator:

Name:	John Zaia
Institution:	City of Hope, Beckman Research Institute
Type:	PI

Disease Focus: Blood Disorders, Blood Cancer, Cancer, HIV/AIDS, Solid Tumor

Human Stem Cell Use: Adult Stem Cell

Award Value: $8,000,000

Status: Active

Progress Reports

Reporting Period: Year 1

View Report

Grant Application Details

Application Title: The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.

Public Abstract:

As the largest provider of bone marrow cell transplants in California, and the second largest in the nation, our institution has great expertise and an excellent record of safety in the delivery of stem cell treatments. We now propose to create the Alpha Clinic for Cell Therapy and Innovation (ACT-I) in which new, state-of-the-art, stem cell treatments for cancer and devastating blood-related diseases will be conducted and evaluated. As these experimental therapies prove to be effective, and become routine practice, our ACT-I Program will serve as the clinical center for delivery of these treatments. ACT-I will be an integral part of our Hematologic Malignancy and Stem Cell Transplantation Institute, placing it in the center of our institutional strengths, expertise, infrastructure and investment over the next decade. To move quickly once the CIRM award is made, ACT-I can be launched within our institution's Day Hospital, a brand new, outpatient blood stem cell transplantation center opened in late 2013 with California Department of Health approval for 24 hour a day operation. This will ensure that ACT-I will have all the clinical and regulatory expertise, trained personnel, state-of-the-art facilities and other infrastructure in place to conduct first-in-human clinical trials and to deliver future, stem cell-based therapies for cancer and blood-related diseases, including AIDS. When our new Ambulatory Treatment Center is complete in 2018, it will double our capacity for patient visits and allow for expansion of the ACT-I pipeline of new stem cell products in a state-of-the-art facility.

Beyond our campus, we operate satellite clinics covering an area that includes urban, suburban and rural sites. More than 17.7 million people live in this area, and represent some of the greatest racial and ethnic diversity seen in any part of the country. Our ACT-I is prepared to serve a significant, diverse and underserved portion of the population of California.

CLINICAL TRIALS. Our proposal has two lead clinical trials that will be the first to be tested in ACT-I. One will deliver transplants of blood stem cells that have been modified to treat patients suffering from AIDS and lymphoma. The second will use neural stem cells to deliver drugs directly to cancer cells hiding in the brain. These studies represent some of the new and exciting biomedical technologies being developed at our institution. In addition to the two lead trials, we have several additional clinical studies poised to use and be tested in this special facility for clinical trials. In summary, ACT-I is well prepared to accommodate the long list of clinical trials and begin to fulfill the promise of providing new stem cell therapies for the citizens of California.

Statement of Benefit to California:	California's citizens voted for the California Stem Cell Research and Cures Act to support the development of stem cell-based therapies that treat incurable diseases and relieve human suffering. To achieve this goal, we propose to establish an Alpha Clinic for Cellular Therapies and Innovation (ACT-I) as an integral part of our Hematological Malignancies and Stem Cell Transplantation Institute, and serve as the clinical center for the testing and delivery of new, cutting-edge, cellular treatments for cancer and other blood-related diseases. Our institution is uniquely well-suited to serve as a national leader in the study and delivery of stem cell therapeutics because we are the largest provider of stem cell transplants in California, and the second largest in the country. According to national benchmarking data, our Hematopoietic Cell Transplantation program is the only program in the nation to have achieved survival outcomes above expectation for each of the past nine years. This program currently offers financially sustainable, research-driven clinical care for patients with cancer, HIV and other life-threatening diseases. CIRM funding will allow the ACT-I clinic to ramp up quickly, drawing upon institutionally established protocols, personnel and infrastructure to conduct first-in-human clinical trials for assessment of efficacy. As CIRM funding winds down, ACT-I will have institutional support to offer proven cellular therapeutics to patients. The lead studies at the forefront of the ACT-I pipeline of clinical trials focus on treatments for HIV-1 infection and brain tumors, two devastating and incurable conditions. These first trials are closely followed by a robust queue of other stem cell therapeutics for leukemia, lymphoma, prostate cancer, brain cancers and thalassemia. Our long list of proposed treatments addresses diseases that have a major impact on the lives of Californians. Thalassemia is found in up to 1 in 2,200 children born in California; prostate cancer affects 211,300 men, and HIV-1 infection occurs in 111,000 of our citizens. From 2008 to 2010, 6,705 Californians were diagnosed with brain cancers, 4,580 of whom died. In considering hematological malignancies during this same period, 2,800 patients were diagnosed with Hodgkin lymphoma (416 died), 20,351 with non-Hodgkin lymphoma (6,241 died), 13,358 with leukemia (6,961 died), 3,900 with acute myelogenous leukemia (2,972 died), 2,129 with acute lymphoblastic leukemia (648 died) and 4,198 with chronic lymphocytic leukemia (1,271 died). Standard of care fails in many cases; mortality rates for patients with hematological malignancies range from 25% to 76%. Successful stem cell therapeutics hold the promise to reduce disease-related mortality while improving disease-related survival and quality of life for the citizens of California, and for those affected by these diseases worldwide.